

FDA Perspective: Current Regulatory Incentives Orphan Drug Provisions

John J. McCormick, M.D.

Deputy Director, FDA Office of Orphan Products Development

Biological Therapeutics for Rare Plasma Protein Disorders

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RARE PLASMA DISORDERS:

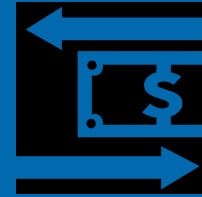
No Profit,
No Drugs



Return on Investment

Total Profit

Cost of Development

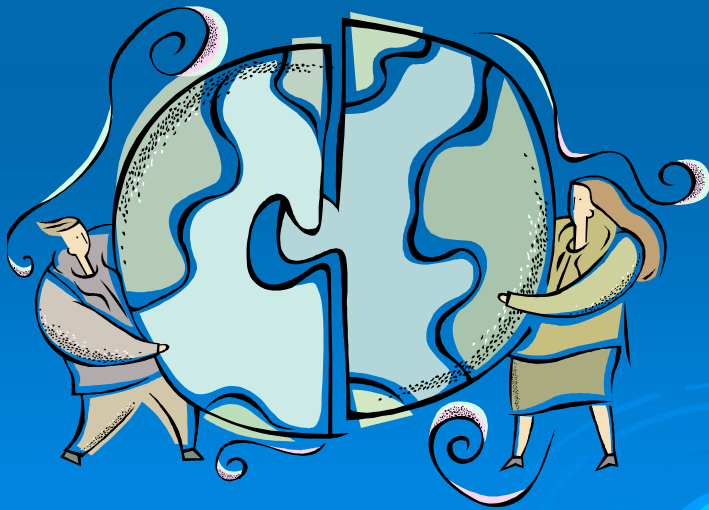


Cost of Development

Basic Research
+
Clinical Research
+
Cost of Money
+
Regulatory Costs
+
Etc.



Each piece of the equation
offers opportunities for
intervention



Profits may be increased by decreasing competition

- Patents
- Exclusivity



Basic Research

- NIH
- Grants
- CRADA



Clinical Research

➤ Grants and Tax Credits

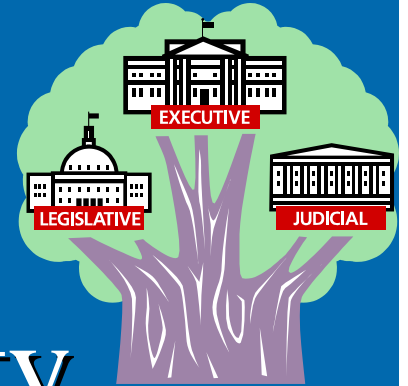
but also

➤ Endpoints and number of Studies

Regulatory Costs

- Fee waiver
- Speed of review
- Need to Know vs. Nice to Know

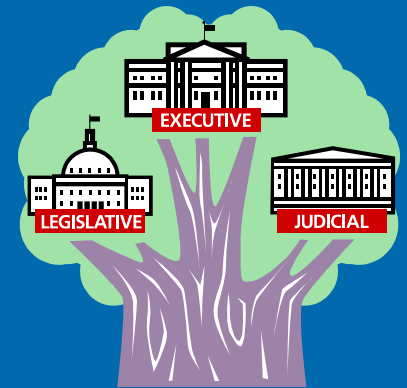
Orphan Drug Act



➤ Seven Years of Exclusivity

- Same drug/same indication
 - Similar to a use patent
- Begins at time of FDA approval

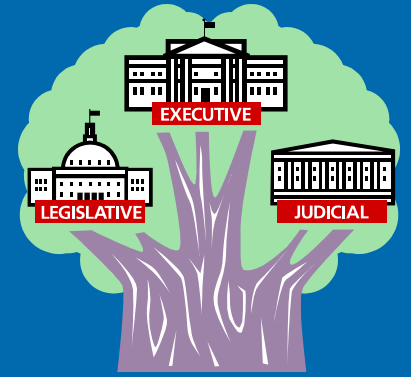
Orphan Drug Act



➤ Tax Credits

- Fifty percent of clinical trial costs.
- Twenty year carry forward; one year carry back.

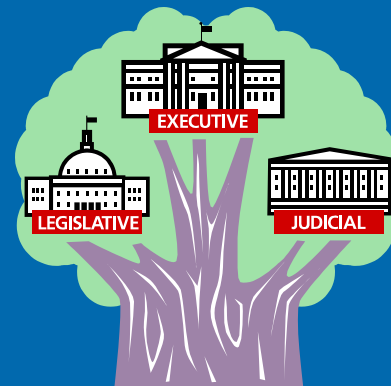
Orphan Drug Act



➤ User Fee Waiver

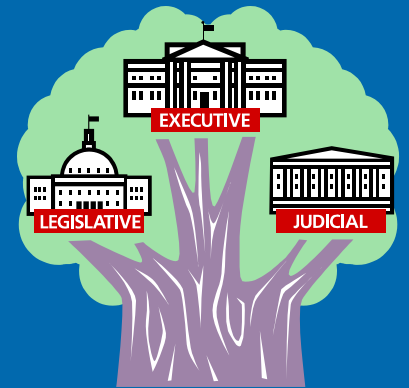
- Application fee only.
- Establishment & product fees not included.

Orphan Drug Act



➤ Written protocol assistance

Orphan Drug Act



➤ Grants

- \$200,000 total cost for Phase I
- \$350,000 total cost for Phase II or III
- Up to three years

OPD Web Site



<http://www.fda.gov/orphan>

Your Link to:

- **Overview of the FDA Office of Orphan Products Development**
- **Guidelines for designation application**
- **List of designated and approved orphan products**
- **Grant application information**
- **List of ongoing orphan grant studies**